Title (en)

METHODS AND COMPOUNDS FOR CURING DISEASES CAUSED BY MUTATIONS

Title (de)

VERFAHREN UND VERBINDUNGEN ZUR BEHANDLUNG VON DURCH MUTATION HERVORGERUFENEN KRANKHEITEN

Title (fr)

PROCEDES ET COMPOSES POUR SOIGNER DES MALADIES CAUSEES PAR DES MUTATIONS

Publication

EP 0906328 A4 20030618 (EN)

Application

EP 97922597 A 19970501

Priority

- US 9707362 W 19970501
- US 64051796 A 19960501

Abstract (en)

[origin: WO9741141A1] The invention concerns the use of duplex oligonucleotides having both 2'-deoxyribonucleotides and ribonucleotides, wherein there is base pairing between the two types of nucleotides. The sequence of the oligonucleotide is selected so that the 3' and 5' most regions of the oligonucleotides are homologous with (identical to) the sequence of a preselected target gene of a cell. The two regions of homology embrace a region that is heterologous with the target sequence. The introduction of the oligonucleotide into the nucleus of the cell causes the alteration of the target gene such that the sequence of the altered target gene is the sequence of the heterologous region. Consequently, the oligonucleotides of the invention are termed chimeric mutation vectors (CMV). In one embodiment of the invention the target gene is a globin gene and the target cell is a hematopoietic stem cell. This embodiment can be used to correct certain hemoglobinopathies such as Sickle Cell Disease, beta -thalassemia and Gaucher Disease. The rate of correction of the globin gene is high enough so that no selection of the treated hematopoietic stem cells is required to obtain a therapeutically significant effect. In one embodiment the ribonucleotides of the CMV contain methylated 2'-O.

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Citation (search report)

- [XD] KMIEC E.B.: "Genomic targeting and genetic conversion in cancer therapy", SEMINARS IN ONCOLOGY, vol. 23, no. 1, 1 February 1996 (1996-02-01), pages 188 - 193, XP000618833
- See references of WO 9741141A1

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